Induced pluripotent stem cells from children with autism spectrum disorders

Grant Award Details

Induced pluripotent stem cells from children with autism spectrum disorders

Grant Type: Tissue Collection for Disease Modeling

Grant Number: IT1-06571

Project Objective: The project objective is to collect biological samples and information from children with Autism for generation of iPSCs as part of the CIRM iPSC initiative.

Investigator:

Name:	Joachim Hallmayer
Institution:	Stanford University
Type:	PI

Disease Focus: Autism, Neurological Disorders, Pediatrics

Award Value: $530,265

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 2

View Report

Reporting Period: NCE Year 3

View Report

Grant Application Details

Application Title: Induced pluripotent stem cells from children with autism spectrum disorders

Public Abstract:

Autism spectrum disorders (ASD) are a family of disabling disorders of the developing brain that affect about 1% of the population. Studying the biology of these conditions has been difficult as they have been challenging to represent in animal models. The core symptoms of ASD, including deficits in social communication, imagination and curiosity are intrinsically human and difficult to model in organisms commonly studied in the laboratory. Ideally, the mechanisms underlying ASDs need to be studied in human patients and in their cells. Since they maintain the genetic profile of an individual, studying neurons derived from human induced pluripotent stem cells (hiPSC) is attractive as a method for studying neurons from ASD patients. hiPSC based studies of ASDs hold promise to uncover deficits in cellular development and function, to evaluate susceptibility to environmental insults, and for screening of novel therapeutics. In this project our goal is to contribute blood and skin samples for hiPSC research from 200 children with an ASD and 100 control subjects to the CIRM repository. To maximize the value of the collected tissue, all subjects will have undergone comprehensive clinical evaluation of their ASD. The cells collected through this project will be made available to the wider research community and should result in a resource that will enable research on hiPSC-derived neurons on a scale and depth that is unmatched anywhere else in the world.

Statement of Benefit to California:

The prevalence and impact of Autism Spectrum Disorders (ASD) in California is staggering. California has experienced 13% new ASD cases each year since 2002. ASD are a highly heritable family of complex neurodevelopmental conditions affecting the brain, with core symptoms of impaired social skills, language, behavior and intellectual abilities. The majority with an ASD experience lifelong disability that requires intensive parental, school, and social support. The result has been a 12-fold increase in the number of people receiving ASD services in California since 1987, with over 50,000 people with ASDs served by developmental and regional centers. Within the school system, the number of special education students with ASD in California has more than tripled between 2002 and 2010. The economic, social and psychological toll is enormous.

It is critical to both prevent and develop effective treatments for ASD. While rare genetic mutations account for a minority of cases, our understanding of idiopathic ASD (>85% of cases) is extremely limited. Mechanisms underlying ASDs need to be studied in human patients and in cells that share the genetic background of these patients. Since they maintain the complete genetic background of an individual, hiPSCs represent a very practical and direct method for investigating neurons from ASD patients to uncover cellular deficits in their development and function, and for screening of novel therapeutics.

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